4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No.FDA-2011-D-0847]

Guidance for Industry and Food and Drug Administration Staff; Humanitarian Use Device (HUD) Designations; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a guidance for the industry and FDA staff entitled "Humanitarian Use Device (HUD) Designations." Devices are eligible for HUD designation if they are designed to treat or diagnose a disease or condition that affects or is manifested in fewer than 4,000 individuals in the United States per year. Devices that receive HUD designations may be eligible for marketing approval under the Humanitarian Device Exemption (HDE) marketing pathway. This guidance document is intended to assist applicants in the preparation and submission of HUD designation requests and FDA reviewers in evaluating such requests. This guidance finalizes the draft guidance of the same title dated December 2011.

DATES: Submit either electronic or written comments on Agency guidances at any time.

ADDRESSES: Submit written requests for single copies of the guidance to the Office of Orphan Products (OOPD), Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 32, rm. 5271, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist the office in processing your requests. The guidance may also be obtained by mail by calling OOPD at

301-796-8660. See the SUPPLEMENTARY INFORMATION section for electronic access to the guidance document.

Submit electronic comments on the guidance to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT:

Eric Chen

Office of Orphan Products Development (OOPD)

Food and Drug Administration

Bldg. 32, rm. 5222

10903 New Hampshire Ave.,

Silver Spring, MD 20993

301-796-6327

email: eric.chen@fda.hhs.gov

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a guidance for industry and FDA staff entitled "Humanitarian Use Device (HUD) Designations." Devices are eligible for HUD designation if they are designed to treat or diagnose a disease or condition that affects or is manifested in fewer than 4,000 individuals in the United States per year. (See section 520(m) of the Federal Food, Drug, and Cosmetic Act (FD&C Act), 21 U.S.C. 360j(m); 21 CFR 814.102.) This guidance document is intended to assist applicants in the preparation and submission of HUD designation

requests to OOPD. This guidance is also intended to assist FDA reviewers in the evaluation and analysis of HUD designation requests.

Topics addressed in this guidance include: (1) demonstrating in HUD designation requests that the device is designed to treat or diagnose a disease or condition that affects or is manifested in fewer than 4,000 individuals in the United States per year; (2) how this demonstration varies depending on whether the device is intended for therapeutic or diagnostic purposes; (3) how properties of the device may affect this demonstration; and (4) for the purpose of a HUD designation request, delineating a medically plausible subset ("orphan subset") of persons with a given disease or condition that affects or is manifested in 4,000 individuals or more in the United States per year.

Devices that receive HUD designation may be eligible for marketing approval under an HDE application. An HDE application is a premarketing application that is similar to a premarket approval (PMA) application in that the applicant must demonstrate a reasonable assurance of safety, but in an HDE application, the applicant seeks an exemption from the PMA requirement of demonstrating a reasonable assurance of effectiveness. A device that has received HUD designation is eligible for HDE approval if, among other criteria, the device will not expose patients to an unreasonable or significant risk of illness or injury and the probable benefit to health from use of the device outweighs the risk of injury or illness from its use, taking into account the probable risks and benefits of currently available devices or alternative forms of treatment. (See section 520(m)(2)(C) of the FD&C Act; 21 CFR 814.104(b)(2).) Although a HUD designation from OOPD is a prerequisite to submitting an HDE application to the Center

for Devices and Radiological Health or the Center for Biologics Evaluation and Research, it does not by itself guarantee approval of the HDE application.

In the Federal Register of December 13, 2011 (76 FR77542), FDA issued for public comment "Draft Guidance for Industry and Food and Drug Administration Staff on Humanitarian Use Devices Designations" dated December 2011. The Agency issued this draft guidance with the aim of assisting sponsors in the preparation and submission of HUD designation requests by, among other things, providing clarity on particular elements of HUD designation requests that had historically caused confusion among sponsors. In particular, the draft guidance focused on the disease or condition that the device treats or diagnoses, population estimates, orphan subsets, device descriptions, scientific rationales, and supporting documentation.

We received several comments on the draft guidance. Most comments appreciated the clarification and explanation provided by the draft guidance. Several comments made recommendations to improve clarity.

FDA is issuing the draft guidance in final form with minor revisions to improve clarity. This guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The guidance represents the Agency's current thinking on HUD designation requests. It does not create or confer any rights for or on any person and does not operate to bind FDA or the public. An alternative approach may be used if such approach satisfies the requirements of the applicable statutes and regulations.

II. Comments

Interested persons may submit either written comments regarding this document to the Division of Dockets Management (see ADDRESSES) or electronic comments to http://www.regulations.gov. It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at http://www.regulations.gov.

III. Electronic Access

Persons with access to the Internet may obtain this guidance document at either: http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm, http://www.fda.gov/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/default.htm, http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm, or http://www.regulations.gov.

Dated: January 18, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

[FR Doc. 2013-01420 Filed 01/23/2013 at 8:45 am; Publication Date: 01/24/2013]